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EMA/188494/2019
Inspections, Human Medicines Pharmacovigilance & Committees Division

Scientific recommendation on classification of advanced therapy medicinal products

Article 17 – Regulation (EC) No 1394/2007

Disclaimer: This document is a summary for public release of a scientific recommendation on classification of advanced therapy medicinal products. The original text adopted by the Committee for Advanced Therapies (CAT) has been redacted to delete commercially confidential information.

The present scientific recommendation refers exclusively to the case as presented to the European Medicines Agency (EMA) without prejudice to future evaluations by the Agency.

It is stressed that the scientific recommendation on advanced therapy classification does not amount to any endorsement of the plausibility of the product, including the mode of action or therapeutic indication(s) claimed by the applicant.

Brief description (or name when available) of the active substance(s)

Human bone marrow derived allogeneic mesenchymal stem cells expressing human alpha-1 antitrypsin.

Brief description of the finished product

Lentivirally transduced mesenchymal stem cells, cryopreserved in cryomedium.

Proposed indication

Treatment of steroid refractory acute Graft-versus-Host-Disease (GvHD), grades II-IV.

EMA/CAT conclusion

The procedure was finalised on 19 November 2018 for the following recommendation.

On the basis that:

- the product contains a biological medicinal product as the active substance;

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- the product is presented as having properties for, or is administered to human beings with a view to treat a disease through the pharmacological, immunological action of its cells;
- the product contains an active substance which contains a recombinant nucleic acid administered to human beings with a view to regulating, repairing, adding a genetic sequence;
- its therapeutic, prophylactic or diagnostic effect relates directly to the product of genetic expression of this sequence,

the EMA/CAT considers that the product falls within the definition of a gene therapy medicinal product, as provided in Article 2(5) of Regulation (EC) 1394/2007.